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DRUG DISCOVERY AND DEVELOPMENT EDITION

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GeneCode.Ltd

The annual listing of 10 companies in Europe that are at the forefront of providing Drug Discovery and Development solutions and impacting the industry in the region

GENECODE

Unveiling a Game-Changer in Parkinson's Treatment

Prof Dr. Mart Saarma,
Senior Partner,
Member of Board of Directors

In neuro healthcare, a transformative era is dawning, promising less invasive solutions for the treatment of neurodegenerative diseases. Conditions like Parkinson's result in the degeneration and death of neurons in the brain and other parts of the body, leading to debilitating symptoms. While current treatments offer symptomatic relief, they fail to slow down the relentless progression of the diseases.

GeneCode, a private research and development company, pioneers novel drugs to protect, regenerate, and restore compromised neurons. This small-molecule-based disease-modifying drug has demonstrated efficiency in vitro, in cultured neurons, and animal models of Parkinson's, offering hope to over 10 million individuals with the disease.

"We hope to take over the whole Parkinson's field and be the first one to offer the disease-modifying and disease-curing treatment," says Prof Dr. Mart Saarma, senior partner and member of the board of directors at GeneCode.

GeneCode's primary focus revolves around developing treatments for neurodegenerative diseases and antiviral therapies. In efforts to combat neurodegenerative and neurological conditions such as Parkinson's, spinal cord injury, Huntington's, motor neuron disease, multiple sclerosis, and Alzheimer's, GeneCode harnesses its expertise in proprietary drug design technologies to craft small-molecule compounds.

Although clinical trials of GDNF protein and gene therapy have shown promise for Parkinson's disease treatment, the challenge is that they require brain surgery, making the treatment expensive and ethically restricted for early-stage patients.

Working on the groundbreaking research conducted on neuroprotective and potentially neurorestorative treatments, GeneCode collaborates internationally with experts from Estonia, the U.S., Finland, France, UK and Germany.

Using medicinal chemistry and pharmacokinetics, renowned specialists from GeneCode and Argobio, a French therapeutics-based biotech company, as well as researchers from the Universities of Tartu and Helsinki and international CROs, are focused on GDNF mimetics and are contributing together toward disease modification and cures rather than mere symptom relief. Their innovative research concentrates on small molecules that can be administered orally or subcutaneously, non-invasively, obviating the necessity for surgical interventions, to halt the progression of neurodegenerative diseases like Parkinson's. Research has enabled to significantly improve the potency of its compound's properties that efficiently slowed down and stopped the degeneration and deaths of the neurons.

Paavo Pilv,
CEO,
Member of Management Board

New compounds being developed by GeneCode and Agrobio are nearly 1000 times more potent, enabling immediate treatment post-diagnosis. This has the potential to preserve more dopamine neurons, address non-motor symptoms, and advance regenerative medicine. GeneCode is using stem cell technology to investigate in culture the ability of novel compounds to protect and rescue human dopamine neurons derived from patients.

Currently, in the preclinical phase, GeneCode plans to select a 'lead compound' and begin IND studies in 2024 and Phase 1 clinical trials in 2025 with complete support from Enterprise Estonia and the European Innovation Council. While its long-term strategy includes developing a Parkinson's disease drug up to Phase 2, the expert team is open to discussions with global pharmaceutical companies before initiating Phase 3, with the intention of generating commercial interest and market attention.

"We have patents in the U.S. and all EU countries," says Paavo Pilv, CEO and member of the management board at GeneCode Ltd. "We have over 200 new compound structures protected in our pending patent applications."

Notably, GeneCode's drug may have applications in other diseases, such as amyotrophic lateral sclerosis,

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retinitis pigmentosa, and more. It is starting IND studies and production, with clinical trials expected in 2025, pending approval from the EMA.

GeneCode utilizes various techniques, including high throughput screening, stem cell-derived human dopamine neurons, advanced chemical methods and novel animal models of diseases. This project combined computer science, medicinal chemistry, neuroscience, molecular biology, pharmacology, and drug development expertise.

With the ultimate goal of modifying diseases through curative solutions, rather than mere symptom management, GeneCode's impact reaches far beyond the confines of existing treatments. Its vision extends to providing accessible and affordable treatments globally, enhancing the quality of life for individuals affected by these conditions. LS